

David J Rowlands AM
Chair, Petitions Committee
National Assembly for Wales
Cardiff Bay
Cardiff, CF99 1NA

14 January 2019

Dear Mr Rowlands,

Petition P-05-797 Ensure access to cystic fibrosis medicine, Orkambi, as a matter of urgency

Thank you for your letter of 7 December 2018 regarding the aforementioned petition.

We have been in discussion with the All Wales Therapeutics and Toxicology Centre (AWTTC) which advises the All Wales Medicines Strategy Group (AWMSG), to find a solution to make our cystic fibrosis medicines available in Wales.

As a next step, we have a discussion with the AWTTC on 23rd January to scope out how we could make submissions for Orkambi (lumacaftor/ivacaftor) and Symkevi (tezacaftor/ivacaftor) to be used in combination with ivacaftor.

Whilst we cannot provide a precise indication of the timescales involved, we can assure you that we are working to expedite our discussions.

As you may know, there are several challenges in the way that standard cost-effectiveness processes assess the value of these types of precision medicines for severe rare genetic conditions. Innovative life-extending medicines that are used to treat chronic conditions for the lifetime of the patient – and for which the full health benefits are accrued over a lifetime – are substantially undervalued when evaluated under the standard methodologies used by NICE in England. We hope that we can discuss with officials in Wales how we can engage constructively on these issues, as we have recently been able to do in Scotland.

Following productive discussions with the Scottish Government, we will submit Orkambi (lumacaftor/ivacaftor) and Symkevi (tezacaftor/ivacaftor) to be used in combination with ivacaftor to the Scottish Medicines Consortium (SMC) for appraisal. If accepted by the SMC for use on the NHS in Scotland, eligible patients in Scotland should have access to these medicines this year. The SMC's orphan medicines process provides important flexibility for evaluating precision medicines. While the SMC considers its recommendation, Vertex and the Scottish Government have reached an agreement that

clinicians could apply for access for lumacaftor/ivacaftor and tezacaftor/ivacaftor in combination with ivacaftor for eligible patients via the Peer Approved Clinical System (PACS) Tier 2 process at a confidential discount. We hope that we can explore a similar agreement in Wales.

It is worth noting that, while we continue to seek full access to our medicines in Wales, Vertex has provided Orkambi (lumacaftor/ivacaftor) at no cost to 25 patients in Wales because of serious medical need.

We will provide further updates to the Committee in due course.

Yours sincerely,

A handwritten signature in blue ink, appearing to read 'MAOL', with a stylized flourish at the end.

Michael Oliver
UK Country Manager
Vertex Pharmaceuticals